OBJECTIVES: Estimating transition probabilities for Markov models is challenging when the effectiveness of the studied intervention is measured using a continuous score, and only aggregate data by treatment are available. We developed a Bayesian calibration method to estimate transition probabilities and applied it in Parkinson's disease (PD). **METHODS:** A previously published Markov model with health states corresponding to Hoehn and Yahr (H&Y) stages was adapted. Patient-level datasets were simulated to replicate results of clinical trials for different drugs, using the UPDRS scale to assess severity, and transition probabilities were estimated from simulated data to provide a reference case. Two calibrations methods were tested for obtaining transition probabilities without patient-level data. Firstly, the Solver tool of Excel was used, with the mean change in UPDRS score and associated variance as targets. Secondly, a Bayesian calibration was implemented in OpenBUGS to estimate the posterior distribution of transition probabilities, assuming the change in UPDRS score has a normal distribution, with observed mean and variance. All other model input parameters were taken from the original model. **RESULTS:** With simulated patient-level data, the incremental cost (IC) was estimated at €-7,015 (95% credibility interval: €-23,953; €5,977) and incremental QALYs (IQ) at 0.455 (0.112; 0.950). With calibration using the Solver tool, there was an infinity of solutions resulting in IC ranging from €-10,141 to €-8,206 and IQ ranging from 0.422 to 0.473. With calibration using OpenBugs, the IC was estimated at €-6,852 (€-24,244; €6,448) and the IQ at -0.448 (0.108; 0.959). **CONCLUSIONS:** Incremental costs and QALYs obtained using the Bayesian calibration and analysis of patient-level data were similarly distributed. Mean results obtained using the Solver tool were comparable, but no statistical distribution around results could be provided. This example suggests that the Bayesian calibration is a valid method to derive transition probabilities from continuous outcome measures.

PATIENT PREFERENCE STUDIES

PP1
A METHODOLOGY FOR PREDICTING THE IMPACT OF COPAYMENTS ON THE UTILIZATION OF HEALTH TECHNOLOGIES

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OBJECTIVES: Copays (or copayments) for health-technologies, such as pharmaceutical products, are used by governments and insurers to prevent moral hazard, reduce unnecessary utilization of resources, and contribute to the cost of health care. Nevertheless they may also reduce access to necessary care, and the financial protection associated with health insurance. In addition, from the perspective of the manufacturer, the introduction of copays may determine unexpected and abrupt falls in demand. The methodology here presented uses data extracted from surveys on product utilization and patient income, prior and after the introduction of copays in various countries and regions, to predict the potential impact of copays on the demand for a pharmaceutical product in a given country or region. **METHODS:** This approach uses multi-level multivariate regressions and a micro-economic model of demand (which assumes maximization of utility and preference independence) to anticipate changes in utilization (and therefore in demand) as a function of the amount of copay charges. Data from a computer simulation were used to test the method. **RESULTS:** A non-linear relationship between copay and drug-consumption, determined by the combined effect of the amount charged and of the average population income was identified, predicting changes in aggregate demand and in income-specific demand. For instance: if we consider two countries both adopting a 1 Euro co-pay charge on a (not easily substitutable) product and whose average income differs by 20%, we expect the demand to be almost 3% lower in the country with the lower income than in the country with the higher income. **CONCLUSIONS:** Under general market assumptions, it is possible to build models that estimate the potential impact of copay charges. These models can be used to help design health policies and market strategies.

PP2
MULTINATIONAL CONSISTENCY OF A DISCRETE CHOICE MODEL IN QUANTIFYING HEALTH STATES FOR THE EXTENDED 5-LEVEL EQ-5D

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OBJECTIVES: To investigate the feasibility of choice experiments for EQ-5D-5L states using computer-based data collection, and to examine the consistency of the estimated parameters values derived after modeling the stated preference data across countries in a multinational study. **METHODS:** Similar choice experiments were executed in Canada, England, The Netherlands, and United States (US). Interactive software was developed to standardize the format of the choice tasks across countries, except for England where face-to-face interviewers were used. The choice task required respondents to choose between two sub-optimal health states. A Bayesian design was used to generate 200 pairs of states that were randomly grouped into 20 blocks. Each respondent completed one block consisting of 10 pairs. A main-effect alternative-specific multinomial probit regression model was used to estimate regression coefficients and to derive values for each health state that capture the relative differences in levels between states. **RESULTS:** In total there were 1775 respondents, at least 400 respondents from each country, who completed 17750 paired comparisons, resulting into 35500 assessed health states. The mean time to perform one choice task was between 29.2 (US) and 45.2 (England) seconds. All regression coefficients were statistically significant, except level 2 for Usual Activities in The Netherlands (p=0.51). Three regression coefficients with illogical ordering were observed (The Netherlands: level 3 Pain/Discomfort, England: Level 3 Usual Activities & Pain/Discomfort). Predictions for the complete set of 3125 EQ-5D-5L states were similar for the four countries. Intra class correlation coefficients between the countries were high: from 0.89 (England vs. US) through 0.99 (Canada vs. US). **CONCLUSIONS:** This proof of concept study indicates that computer-based choice tasks for the EQ-5D-5L in the general population are feasible and parameter of the choice tasks estimates are generally consistent and logical, and the estimated values are largely consistent between the 4 countries.

PP3
CAN THE USE OF SOCIAL MEDIA AND MOBILE APPS IMPROVE PATIENT KNOWLEDGE OF DISEASE AND HEALTH OUTCOMES? A SYSTEMATIC REVIEW

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OBJECTIVES: The use of interactive social media (SM) based on Web 2.0 technology, e.g. Twitter, Facebook, MySpace; and mobile apps, is increasing but its role in improving patient knowledge of their disease and its management is unclear. We conducted a systematic review to assess the evidence for health benefits from SM. **METHODS:** We searched MEDLINE and EMBASE for relevant articles published in the last five years that used words synonymous with SM; patient, carer, or parents' preferences, opinions or views; pharmacological interventions; and disease. We included comparative studies or systematic reviews on the use of any SM by patients, that measured differences in knowledge about disease, uptake of pharmacological interventions, or clinical outcomes from better management of disease. Articles were excluded if they reported only the use of SM by health care professionals, or if they were case studies, narrative reviews or expressed expert opinions. **RESULTS:** We identified 3,232 unique abstracts, 24 of which reported the use of interactive, internet-delivered programs (n=13), Facebook (n=4), and mobile apps (n=3) , for improving health outcomes of patients with cancer, or inflammatory, mental health, musculoskeletal, neurologic, ophthalmologic, or sexual health-related disorders. Patients receiving SM-based interventions showed improved knowledge of their disease, and better clinical outcomes compared with controls. Two additional studies reported on the use of SM aimed at increasing knowledge, and self-efficacy in parents of children with cystic fibrosis. Overall, the studies showed that SM-based interventions improved knowledge of disease and clinical outcomes compared with control groups. **CONCLUSIONS:** Surprisingly little research has been conducted on the value of SM to aid and support patients. What evidence exists suggests that SM tools offer health benefits. fFrther work is needed to confirm these effects and to assess how best the tools might increase patients' knowledge about disease, treatment adherence and clinical outcomes.

PP4
THE IMPORTANCE OF PATIENT REPORTED OUTCOMES IN REIMBURSEMENT OF ORPHAN PRODUCTS IN EUROPE

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OBJECTIVES: To determine the importance of patient reported outcomes (PROs) in the reimbursement of orphan drugs in Europe and identify any HTA authority preferences for particular PRO measures. **METHODS:** All 31 products assigned an orphan designation by the European Medicines Agency (EMA), between January 2009 and May 2013, were evaluated against the following criteria: approval for marketing, submission for reimbursement/price negotiation to NICE, SMC, G-BA, and HAS, presence or absence of PROs, approval or rejection of reimbursement application. Where available, HTA guidance documents resulting from the applications were reviewed in detail to determine the impact of PRO measures on the reimbursement decision. **RESULTS:** Of the 31 products assigned an orphan designation, 26 were granted marketing authorisation by the EMA. Eleven products were submitted to NICE for reimbursement in England of which 7 submissions contained PROs and 6 were approved. In Scotland, 11 products were submitted for reimbursement to the SMC and of the 7 submissions containing PRO data, 3 were recommended for reimbursement. One submission is still pending. In Germany, 7 products were submitted to the G-BA of which 5 contained PROs and were allowed to enter into price negotiations. The French HTA authority, HAS, evaluated 20 submissions of which 9 contained PROs and 19 were approved. **CONCLUSIONS:** The results of our assessment of EMA approved orphan drugs indicates that a great deal of variation exists across Europe with respect to the evaluation of orphan drugs for reimbursement or price negotiation. In some countries, reimbursement is largely independent of evidence of reported patient benefit while in others, where evidence of economic value is critical for success, robust PRO data are essential.

RESEARCH PODIUM PRESENTATIONS – SESSION II
RESEARCH ON METHODS – CLINICAL STUDIES

CL1
A COMPARISON OF METHODOLOGIES FOR ESTIMATING SURVIVAL IN PATIENTS TREATED WITH SECOND-GENERATION TYROSINE-KINASE INHIBITORS (TKIS) FOR CHRONIC MYELOID LEUKAEMIA (CML)

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OBJECTIVES: NICE have previously recommended nilotinib, but not dasatinib, as a first-line (TA251) and second-line (TA241) treatment in chronic phase (CP) CML. Within these appraisals, different methods were used to estimate overall survival (OS). Bosutinib, a potent dual Src/Abl TKI, is undergoing a NICE appraisal in second-line or later CML (ID495). The objective is to review the OS methods used and assess their impact on cost-effectiveness and recommendations in CML appraisals. **METHODS:** We identify the methodologies used for estimating OS in TA241 and TA251 and investigate the impact of using these to estimate OS for the TKIs, including bosutinib, in relapsed/refractory CML. Finally, we consider the implications of the various methodologies on the cost-effectiveness results and recommendations in previous and future NICE assessments of TKIs for CML. **RESULTS:** The base-case in TA241 used a surrogate relationship between response (MCyR) and OS